



### Targeted off-the-shelf immunotherapy to treat refractory cancers

# **Grant Award Details**

Targeted off-the-shelf immunotherapy to treat refractory cancers

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-09615

Project Objective: To use human induced pluripotent stem cells (iPSCs) to produce a novel targeted, "off-the-shelf"

cellular immunology product that can be translated to clinical therapies to treat patients with

relapsed/refractory cancers that do not currently have an effective treatment option.

Investigator:

Name: Dan Kaufman

Institution: University of California, San Diego

Type:

Disease Focus: Blood Cancer, Cancer, Solid Tumor

Human Stem Cell Use: iPS Cell

Award Value: \$1,936,936

Status: Active

## **Grant Application Details**

Application Title: Targeted off-the-shelf immunotherapy to treat refractory cancers

#### **Public Abstract:**

#### **Research Objective**

This project will use human pluripotent stem cells to produce a standardized, off-the-shelf immunotherapy using novel immune cells that are specifically targeted to cure otherwise lethal cancers.

#### **Impact**

Unlike current immunotherapies produced on a patient-specific basis, iPSC-derived immune cells are targeted to tumors with high specificity, no off-target effects and without need for patient matching

#### **Major Proposed Activities**

- Produce novel human iPSC-derived natural killer (NK) cells expressing a specifically designed receptor (CAR) to more effectively and efficiently target and kill human ovarian and other cancer cells.
- Engineer novel NK cell-specific CAR constructs with NK cell-specific intracellular signaling domains to enhance iPSC-NK cell activation upon recognition of ovarian cancer target cells.
- Optimize anti-leukemia activity of iPSC-derived NK cells using defined iPSC-NK cell
  populations with differing receptor profiles that target specific tumor profiles.
- Optimize iPSC-NK cell expansion using systems that can be rapidly translated into clinical cell production. This will ensure we efficiently produce enough targeted iPSC-NK cells for clinical trials

# Statement of Benefit to California:

Cancer remains the second leading cause of death in California, the US and worldwide. Despite advances in many areas of cancer treatment including chemotherapy and even immunotherapy, outcomes for relapsed or refractory cancers remain dismal. Here, we will use human iPSCs as the basis to develop a novel targeted cell population that can serve as a universal cellular immunotherapy to better treat and cure cancers for potentially thousands of patients in California each year.

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